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## Hematopoietic Stem Cell Gene Therapy for X-linked Agammaglobulinemia

### Grant Award Details

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Hematopoietic Stem Cell Gene Therapy for X-linked Agammaglobulinemia

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-12111

**Project Objective:** To develop an autologous, gene-modified, HSC cell therapy candidate for X-linked Agammaglobulinemia (XLA)

**Investigator:**

<b>Name:</b>	Donald Kohn
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

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**Disease Focus:** Blood Disorders

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$219,230

**Status:** Active

### Grant Application Details

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**Application Title:** Hematopoietic Stem Cell Gene Therapy for X-linked Agammaglobulinemia

**Public Abstract:****Research Objective**

The objectives of this study are to advance a stem cell gene therapy for the immunodeficiency XLA, defining the final therapeutic candidate and showing therapeutic activity in a relevant mouse model.

**Impact**

XLA can be treated with chronic immunoglobulin replacement, but may be sub-optimal due to infections and inflammatory complications. Stem cell gene therapy may provide a curative one time treatment.

**Major Proposed Activities**

- 1. Assess BTK lineage expression and humoral immune reconstitution in BTK/TEK double knock-out mouse model of XLA by BTK gene editing and transplantation to demonstrate disease modifying activity.
- 2. Compare different BTK transgene expression units for the lineages, levels and lymphocyte function they produce to define optimal candidate.
- 3. Assess safety of BTK editing by secondary transplants of edited cells into congenic (CD45.1) recipients.
- 4. Establish draft Target Product Profile.
- 5. Develop measures of identity, activity and purity.
- 6. Define therapeutic candidate, based on results of above studies

**Statement of Benefit to California:**

Regenerative medicine methods using genetically-corrected human stem cells will result in novel, effective therapies for blood cell diseases to improve the health of millions of Californians and tens of millions of people world-wide. Scientific findings and biomedical materials produced will be publicly available to non-profit and academic organizations in California, and any intellectual property developed by this Project will follow the guidelines of CIRM to benefit the State of California.

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